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CRITICAL ASSESSMENT OF BELGIAN REIMBURSEMENT DOSSIERS OF ORPHAN DRUGSDenis A¹, Mergaert L¹, Fostier C¹, Cleemput I², Hulstaert F², Simoens S³¹Yellow Window Management Consultants, Antwerp, Belgium; ²Belgian Health Care Knowledge Centre, Brussels, Belgium; ³K.U. Leuven, Leuven, Belgium

OBJECTIVES: This study aims to conduct an assessment of reimbursement dossiers of orphan drugs in Belgium. First, a qualitative analysis reviews the evidence submitted in reimbursement dossiers of all orphan drugs. Second, an in-depth analysis compares the clinical evidence submitted to the European Medicines Agency with the Belgian reimbursement dossiers for selected orphan drugs. **METHODS:** A qualitative analysis examined all reimbursement dossiers of orphan drugs that have been submitted in Belgium between January 2002 and June 2008. The following information was extracted from each dossier: description of the orphan drug; indication; reimbursement status; therapeutic value and needs; budget impact; and number of registered indications. For selected orphan drugs, an in-depth analysis extracted and compared information about the clinical trials, their primary endpoints and results from the following data sources: European Medicines Agency documents (i.e. the marketing authorization application file, the European Public Assessment Report and the Summary of Product Characteristics); and the Belgian reimbursement dossiers. **RESULTS:** Reimbursement was awarded to the majority of orphan drugs. In addition to the official criteria, other arguments such as the price, employment, patient population, funding of diagnostic tests by the company seemed to play a role in the reimbursement decision. Despite the low number of patients, randomized controlled trials were conducted for many orphan drugs. Budget impact analyses were simplistic and did not consider the impact across multiple indications. Also, some differences were observed in the clinical evidence submitted to the European Medicines Agency and the Belgian reimbursement authorities. **CONCLUSIONS:** There is substantial variation in the evidence that is submitted to the Belgian authorities in the context of an orphan drug reimbursement dossier. Also, some differences have been noted in the clinical evidence reported in European Medicines Agency documents and the evidence included in Belgian reimbursement dossiers of orphan drugs.

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DOES HUMAN MEDICINES DEVELOPMENT IN THE EUROPEAN UNION ADDRESS GLOBAL AND REGIONAL HEALTH CONCERNS?Catalá-López F¹, García-Altés A², Álvarez-Martín E³, Génova-Maleras R⁴, Morant-Ginestar C⁴¹Spanish Medicines and Healthcare Products Agency (AEMPS), Madrid, Spain; ²King's College London, London, UK; ³Rey Juan Carlos University, Madrid, Spain; ⁴Regional Health Council of Madrid, Madrid, Spain

OBJECTIVES: Our aim was to investigate whether efforts to develop innovative medicines in the European Union (EU) are focusing on the most relevant conditions from a global public health perspective. **METHODS:** We reviewed the information on new medicinal products approved by centralised procedure (from 1995 to 2009), available for the public in the European Commission Register of medicinal products and the European Public Assessment Reports (EPAR) from the European Medicines Agency. We included morbidity-mortality data for each disease group, according to the World Health Organization Global Burden of Disease project. We evaluated the association between authorised medicinal products and burden of disease measures (disability-adjusted life-years [DALYs]) in the EU and worldwide. **RESULTS:** We considered 520 marketing authorizations for medicinal products and 338 active ingredients. New authorizations were seen to increase over the period analyzed. There was a positive, high correlation between DALYs and new medicinal products development ($p = 0.619$, $P = 0.005$) in the EU, that was moderate for middle-low income countries ($p = 0.497$, $P = 0.030$) and worldwide ($p = 0.490$, $P = 0.033$). In the EU, the most neglected conditions (related to their attributable health losses) were neuropsychiatric diseases, cardiovascular diseases, respiratory diseases, sense organ conditions or digestive diseases, while globally they were perinatal conditions, respiratory infections, sense organ conditions, respiratory diseases, or digestive diseases. **CONCLUSIONS:** Our findings suggest that the development of new medicinal products is higher in some diseases than in others. Pharmaceutical industry and decision-makers are invited to consider the implications of this imbalance establishing work plans that allow for setting future priorities from a public health perspective.

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DEVELOPMENT OF SYSTEMATIC PROCESS MAPS TO ENABLE COMPARISON BETWEEN DIFFERENT HTA AND DECISION MAKING SYSTEMS: THE FIRST STEP TO BENCHMARKINGPichler FB¹, Allen N², Salek S², Liberti L¹¹CMR International Institute for Regulatory Science, London, UK; ²Cardiff University, Cardiff, UK

OBJECTIVES: The current variability in European health technology assessment and decision-making leads to uncertainty about how to measure efficiency and hampers adoption of new best-practice initiatives, such as the EUnetHTA Joint Action. In addition, such variability in organization makes comparison of the activities and outcomes of these systems problematic. The objective of this study was to develop a systematic process-mapping methodology to both clarify how these systems were organised and to enable comparison between the systems. **METHODS:** The process maps were designed from the point of view of the path that a new medicine takes from market authorization to market access. Key decision and evaluation gateways

occurring within the process and key activities, such as provision of scientific advice, were overlaid onto the process maps to further illuminate and contrast the different systems. **RESULTS:** The systems of eleven countries were mapped and common processes identified. Considerable differences in the systems were detected, including the extent of independence of appraisal, pricing and decision making bodies. However, despite differences in organization and sequence, the general path that a new medicine undertakes from registration to reimbursement was broadly similar. **CONCLUSIONS:** The process maps are a useful tool for the comparative visualization of the process of registration to reimbursement for new medicines. This methodology enables the identification of milestones that can be used to establish a benchmarking programme for the purpose of comparative performance evaluation between different countries. It is envisaged that benchmarking of HTA and decision-making agencies will lead to both process improvement and increased understanding of the costs and benefits of making changes in the systems under evaluation—as has been the case for our previous activities benchmarking the drug regulatory authorities.

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COMPARISON OF THE USE OF PRESCRIBED MEDICATIONS BETWEEN QUEBEC RESIDENTS COVERED BY PUBLIC AND PRIVATE DRUG INSURANCE: THE REMED REGISTRYBlais L¹, Cyr MC¹, Perreault S¹, Moisan J², Bérard A¹, Lalonde L¹, Rahme E³¹Université de Montréal, Montréal, QC, Canada; ²Laval University, Québec, QC, Canada;³McGill University Health Centre, Montreal, QC, Canada

OBJECTIVES: To describe the use of medications among Quebec (Canada) residents covered by private drug insurance and to compare with Quebec residents covered by the public drug insurance plan. **METHODS:** Persons aged less than 65 years with private drug insurance were recruited in community pharmacies and medical clinics between 2007 and 2010 and were included in a computerized registry called reMed. Patient's identification, private drug plan information and socio-demographic variables were gathered at recruitment. Data related to prescriptions dispensed in community pharmacies are transferred to reMed bi-monthly from the community pharmacies' computer services providers. We compared the 10 most prevalent medication classes dispensed (using the AHFS classification) and the average drug cost between reMed participants and Quebec residents covered by the public drug insurance plan. **RESULTS:** In 2008, 2 211 reMed participants filled at least one prescription in a community pharmacy. Participants were 43 years old on average, 31% were male, 19% were current smokers and 46% were overweight or obese. In 2008, analgesics/antipyretics, psychotherapeutic agents and antileptic agents were the three most dispensed classes of medications among reMed participants and Quebec residents with public drug insurance. Despite that 8 of the 10 most prevalent classes of medications were in common between patients with public and private drug insurance, the average cost of medications was 22% lower among patients with public drug insurance (\$37.92 versus \$46.42 per prescription). **CONCLUSIONS:** Patients with private and public drug insurance were found to have a similar distribution of medications dispensed in community pharmacies. However, we found that patients covered by the public drug insurance plan paid on average their medications 22% less than patients covered by a private drug insurance plan.

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THE COLLECTION AND USE OF "REAL WORLD" DATA: INDUSTRY VIEWSDevlin N¹, Peperell K², Gillen D³, Radway-Bright EL⁴¹Office of Health Economics, London, UK; ²pH Associates, Marlow, UK; ³Pfizer, Surrey, UK;⁴Association of the British Pharmaceutical Industry, London, UK

OBJECTIVES: 'Real world' data can improve understanding of the cost-effectiveness of health care technologies and are likely to become increasingly important in decisions affecting patient access and in drug development. However little is known about the investment industry currently makes in these studies, or their impact on HTA. The aim of this study was to examine industry views on the relevance and impact of 'real world' data on HTA and on the relative advantages and disadvantages of conducting these studies in the UK. **METHODS:** Survey of British pharmaceutical companies. **RESULTS:** Responses were received from 31 British pharmaceutical companies, covering most of the larger companies in terms of market share. Three quarters of these reported they were engaged in collecting real life data of some kind in the UK. Data collection efforts were more common for health service costs, resource use and treatment pathways; clinical outcomes; burden of disease; and patient outcomes data. The collection of data on patients' ability to work; patient costs; and impacts on patients' caregivers/family was less common. The availability of local expertise on HTA was a leading factor in choosing to undertake such studies in the UK; other important factors were the perceived importance of local data to NICE; and the influence of NICE on HTA decisions in other markets, particularly elsewhere in Europe. The main barrier to conducting studies in the UK was cost and perceived difficulty in obtaining ethics consent and NHS approval. Views on whether 'real world' evidence influenced NICE decisions were somewhat equivocal. However, participants felt that real world data were likely to be more important in providing evidence on health outcomes in chronic conditions, to track outcomes over longer time periods than is feasible in clinical trials, and for cancer and 'orphan' medicines. **CONCLUSIONS:** While industry recognises the value of 'real world' evidence, there are barriers to conducting such studies in the UK.